

Acute myeloid leukaemia: tests, treatment by setting, options and problems during treatment

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Updated 17 October 2026 · No doses · Verify current approvals and product information

A. Diagnosis and work-up tests and what they change

Work-up of suspected AML: what each test adds

Blood

- CBC, smear review
- PT, aPTT, fibrinogen
- Metabolic panel, LDH, urate, phosphate
- Flow on blood if many blasts

Marrow

- Aspirate (500-cell count) + biopsy
- Flow cytometry: lineage
- Karyotype (banding) ± FISH
- Tell the lab before the procedure

Genes

- FLT3 (ITD + TKD), NPM1, IDH1/2: fast
- Myeloid NGS panel incl. TP53, CEBPA
- PML::RARA by PCR or FISH if APL
- Germline clues: DDX41, RUNX1, GATA2

The patient

- HLA type patient and siblings
- Echo before anthracycline
- HBV, HCV, HIV; pregnancy test
- Fertility advice, central line

CT or MRI of the brain and a lumbar puncture only for neurological symptoms (correct platelets and clotting first; defer the LP in APL until the coagulopathy is corrected). PET-CT only for suspected myeloid sarcoma. Results that change induction (FLT3, CBF fusions, APL) should be available within days.

Work-up (ELN 2022 recommendations, Döhner 2022; UpToDate AML diagnosis and induction reviews).

Table A. Diagnosis and work-up tests and what they change

Test	When	What it changes	Pitfalls
CBC + smear review	Every suspected case	Blasts, Auer rods, abnormal promyelocytes (APL)	A normal or low white count does not exclude AML
PT, aPTT, fibrinogen, D-dimer	Every case, at once	DIC; APL suspicion; transfusion targets	Recheck often in APL
Potassium, phosphate, calcium, urate, creatinine, LDH	Before and during induction	Tumour lysis risk and prevention	Pseudohyperkalaemia and false low glucose/oxygen with very high counts
Marrow aspirate + biopsy; flow cytometry	All (blood alone may do if many blasts)	Myeloid lineage; blast %; baseline for MRD	Tell the laboratory first; sternum for aspirate only
Karyotype ± FISH	All	CBF, KMT2A, MECOM, complex/monosomal: ELN risk	FISH does not replace banding

Test	When	What it changes	Pitfalls
FLT3, NPM1, IDH1/2 (rapid); NGS myeloid panel	All	Midostaurin/quizartinib; ELN risk; targeted drugs	Repeat at relapse; germline variants may show up
PML::RARA by PCR or FISH	Suspected APL	Confirms APL; baseline for molecular remission	Do not wait for it to start ATRA
HLA typing (patient and siblings)	Fit patients; intermediate/adverse risk	Donor search early	Exclude a familial disorder before using a sibling donor
Echo; HBV, HCV, HIV; pregnancy test	Before induction	Anthracycline choice; antiviral prophylaxis	Weak heart: non-anthracycline induction
Brain imaging + lumbar puncture	Neurological symptoms only	CNS disease → intrathecal therapy ± RT	Correct platelets and clotting first; defer in APL

B. Treatment by setting

Induction for fit adults: one backbone, add-ons chosen by the genes

7+3: seven days of cytarabine with an anthracycline on days 1–3 (daunorubicin or idarubicin)

FLT3 mutated

- Add midostaurin (ITD or TKD)
- or quizartinib (ITD)
- RATIFY · QuANTUM-First

CBF, CD33+

- Add gemtuzumab ozogamicin
- t(8;21) or inv(16)
- Meta-analysis: fewer relapses

t-AML / AML-MR

- CPX-351 (liposomal daunorubicin + cytarabine)
- Then transplant if fit

Other paths

- Comorbid: venetoclax + HMA
- Weak heart: no anthracycline
- Sarcoma: add RT ~20 Gy

Day 14–21 marrow (nadir)

Hypoplastic with <5% blasts → wait for counts

Day ~28 marrow (recovery)

CR: <5% blasts, no blasts in blood; MRD status

Pregnancy: first trimester → termination, then treat; later → discuss urgent induction versus temporary measures.
Azacitidine-venetoclax gave more composite remissions and more transplants than 7+3 in a randomised trial (Fathi 2026) but not longer survival; most experts await more data.

Induction (Stone 2017; Erba 2023; Hills 2014; Lancet 2018; Fathi 2026; UpToDate induction and therapy-related neoplasm reviews). Regimen names only, no doses.

Table B. Treatment by setting

Setting	Usual approach	Alternatives	Key points
Fit, FLT3 wild type	7+3 (cytarabine + daunorubicin or idarubicin)	Venetoclax + HMA in selected intermediate/adverse risk	Day 14–21 nadir marrow; remission marrow ~day 28
Fit, FLT3 mutated	7+3 + midostaurin (ITD or TKD) or quizartinib (ITD)	—	Continue the inhibitor in consolidation and maintenance
Fit, CBF AML (CD33+)	7+3 + gemtuzumab ozogamicin	—	Follow fusion transcripts (MRD) by PCR
Therapy-related or MDS-related AML	CPX-351, then allogeneic HCT	Venetoclax + HMA if less fit	Five-year OS 18% vs 8% with 7+3
In CR: favourable risk	Intermediate-dose cytarabine consolidation	HCT if MRD persists	MRD guides
In CR: intermediate risk	Consolidation or allogeneic HCT	Oral azacitidine maintenance (≥55)	MRD-positive → transplant
In CR: adverse risk	Allogeneic HCT in first CR	Consolidation if no donor	Start donor search at diagnosis

Setting	Usual approach	Alternatives	Key points
Unfit, not frail	Venetoclax + azacitidine (or decitabine)	IDH1: ivosidenib + azacitidine; HMA alone; low-dose cytarabine	Marrow day 14–21 of cycle 1; adjust venetoclax with azoles
Frail	Supportive care	Gentle therapy if the patient wishes	Transfusions, antimicrobials, symptom control at home
APL, WBC ≤10,000	ATRA + arsenic trioxide	ATRA + anthracycline if no ATO	ATRA on suspicion; no maintenance after ATO-based CR
APL, WBC >10,000	ATRA + anthracycline, or ATRA + ATO with cyto-reduction	—	Highest risk of bleeding and differentiation syndrome
Relapsed or refractory	Targeted drug by mutation, or salvage chemotherapy	Venetoclax + HMA; trial	Allogeneic HCT is the only cure

C. Treatment options: evidence and best fit

Relapsed or refractory AML: retest the genes, look for a donor, aim for transplant

FLT3

- Gilteritinib
- OS 9.3 vs 5.6 months vs salvage (ADMIRAL)

IDH1 / IDH2

- Ivosidenib or olutasidenib (IDH1)
- Enasidenib (IDH2)
- Watch for differentiation syndrome

NPM1 / KMT2A

- Menin inhibitors: revumenib, ziftomenib
- FDA: NPM1 in Oct–Nov 2025
- India: availability to confirm

No target

- Salvage chemotherapy (FLAG-IDA, MEC)
- or venetoclax + HMA
- Clinical trial

Primary refractory disease affects 20–50% after intensive induction. Only allogeneic HCT (or, after a transplant, a second HCT or donor lymphocytes) can cure relapsed AML; type HLA and call the transplant team when relapse is found. Survival after HCT ranges from about 40% to under 5% depending on remission duration, genetics and disease at transplant.

Relapsed disease (Perl 2019; DiNardo 2018; Stein 2017; FDA notices 2025; UpToDate relapsed AML review).

Table C. Treatment options: evidence and best fit

Option	Best fit	Evidence	Watch for
Midostaurin + 7+3	FLT3-ITD or TKD, fit	RATIFY : 4-year OS 51% vs 44%	Nausea, mucositis
Quizartinib + 7+3	FLT3-ITD, fit	QuANTUM-First : median OS 32 vs 15 months	QT prolongation, neutropenia
Gemtuzumab ozogamicin	CD33+ CBF AML	Hills 2014 : fewer relapses, better OS (fav/int)	Liver sinusoidal obstruction
CPX-351	t-AML, AML-MR, 60–75 years	Lancet 2021 : 5-year OS 18% vs 8%	Long cytopenias
Oral azacitidine maintenance	≥55 years, intermediate/adverse, in CR	QUAZAR : OS 25 vs 15 months	Cytopenias, nausea
Venetoclax + azacitidine	Unfit adults	VIALE-A : OS 15 vs 10 months; CR+CRi 65% vs 23%	Febrile neutropenia, TLS at start
Ivosidenib + azacitidine	IDH1-mutated, unfit	AGILE : OS 24 vs 8 months	Differentiation syndrome, QT prolongation
Gilteritinib	Relapsed FLT3-mutated	ADMIRAL : OS 9.3 vs 5.6 months	Differentiation syndrome, liver tests
Ivosidenib / olutasidenib; enasidenib	Relapsed IDH1; IDH2	DiNardo 2018 ; Stein 2017	Differentiation syndrome
Revumenib; ziftomenib	Relapsed NPM1 (revumenib also KMT2A)	FDA 2025 : CR+CRh 21% (ziftomenib)	Differentiation syndrome, QT; India availability to confirm
ATRA + arsenic trioxide	APL, low/intermediate risk	APLO406 : 72-month EFS 97% vs 77%	Differentiation syndrome, QT, liver
Allogeneic HCT	Adverse risk, MRD-positive, relapse	Koreth 2009 : benefit in intermediate/adverse risk	GVHD, infection, transplant mortality

D. Managing problems during treatment

Infection: prevent during neutropenia, treat fever as an emergency

Prevent

- Fluoroquinolone while ANC <500 (expected >7 days)
- Fluconazole from start of induction
- Second induction or no response: mould-active azole
- Aciclovir if HSV-positive

Fever = emergency

- Cultures, then antibiotic within 60 minutes
- Cefepime, meropenem or piperacillin-tazobactam
- Vancomycin only for line, skin, pneumonia or shock

Still febrile

- Stable: do not change for fever alone
- Febrile after 4–7 days: search for fungus
- Add an antifungal
- Right-sided pain: typhlitis

India: in an Indian tertiary cancer centre, multidrug-resistant organisms grew in 55% and fungal pneumonia was diagnosed in 55% of patients during induction despite prophylaxis; induction mortality 7.4% (Jain 2020). Choose empirical antibiotics from your own resistance data.

Infections (Freifeld 2011; Taplitz 2018; Bucaneve 2005; Cornely 2007; Jain 2020; UpToDate neutropenic fever and prophylaxis reviews).

Table D. Managing problems during treatment

Problem	Prevent	Recognise	Act
Neutropenic fever	Fluoroquinolone; azole; hand hygiene	Temperature ≥38 °C with ANC <500	Cultures; antipseudomonal beta-lactam within 60 minutes
Invasive mould infection	Mould-active azole if second induction	Fever after 4–7 days of antibiotics; lung nodules	Antifungal therapy; look for the source
Tumour lysis	Fluids; allopurinol or rasburicase by risk	Rising K, PO4, urate, creatinine; low Ca	Fluids, K-lowering, dialysis; no Ca for asymptomatic low Ca
Leukostasis	Start cytoreduction early	Breathless, hypoxic, confused, visual change	Induction or hydroxyurea; leukapheresis if symptomatic
DIC / APL bleeding	ATRA early; platelets and cryoprecipitate	Low fibrinogen, oozing, headache	Keep platelets ≥30–50,000 and fibrinogen >150; no heparin
Differentiation syndrome	Watch weight, oxygen, creatinine daily	Fever, dyspnoea, oedema, effusions, hypotension	Dexamethasone at once; pause drug only if severe
Typhlitis	Early imaging if abdominal pain	Right-sided pain, distension, diarrhoea at nadir	Supportive care, broad antibiotics; surgery if perforation
Anthracycline heart damage	Baseline echo and ECG	Breathlessness, oedema	Echo; cardiology; non-anthracycline options
CNS disease	Not routine prophylaxis in adults	Headache, cranial nerve palsy	Intrathecal therapy; RT for deficits or masses
QT prolongation	ECG; correct K and Mg	Prolonged QTc on ECG	Hold arsenic, quizartinib, ivosidenib or menin inhibitor; correct electrolytes